LADICell

Grant Award Details

LADICell

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-11480

Project Objective: To **develop** an ex-vivo autologous gene therapy approach for Leukocyte Adhesion Deficiency-I (LAD-I)

Investigator:

Name:	Kinnari Patel
Institution:	Rocket Pharmaceuticals, Inc.
Type:	PI

Disease Focus: Blood Disorders, Leukocyte Adhesion Deficiency

Human Stem Cell Use: Adult Stem Cell

Award Value: $6,567,085

Status: Pre-Active

Grant Application Details

Application Title: LADICell

Public Abstract:

Therapeutic Candidate or Device

The therapeutic candidate is an ex-vivo autologous gene therapy approach for Leukocyte Adhesion Deficiency-I (LAD-I).

Indication

The target clinical indication is Leukocyte Adhesion Deficiency-I (LAD-I), a pediatric hematological rare disease.

Therapeutic Mechanism

The therapeutic is based in an ex-vivo, lentiviral-based, patient-specific approach by genetically engineering the patient's own CD34 positively selected cells with the corrected genetic sequence of ITGB2 (aka LAD-I) gene and subsequently infusing the corrected cells to deliver a potential cure.

Unmet Medical Need

Children with severe LAD-I present with recurrent, life-threatening infections resulting in ~60-75% mortality prior to reaching the age of 2 years in the absence of a successful allogeneic HSCT (due to extensive bacterial or fungal infection). LADICell will treat the underlying gene defect.

Project Objective

Phase 2 trial completed

Major Proposed Activities

- Patient recruitment, screening, and support (by various CMOs) on their clinical journey
- Enrollment of patients at UCLA
- Cell processing in California

Statement of Benefit to California:

Cell processing activities in California provides jobs and an opportunity to be at the forefront of gene therapy to Californian workers. Clinical studies at UCLA Mattel Children's Hospital will further provide that medical and scientific community premier access to a cutting-edge gene therapy trial.

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